



Optimizing delivery of ATMPs in the healthcare systems: Viewpoint from a healthcare professional

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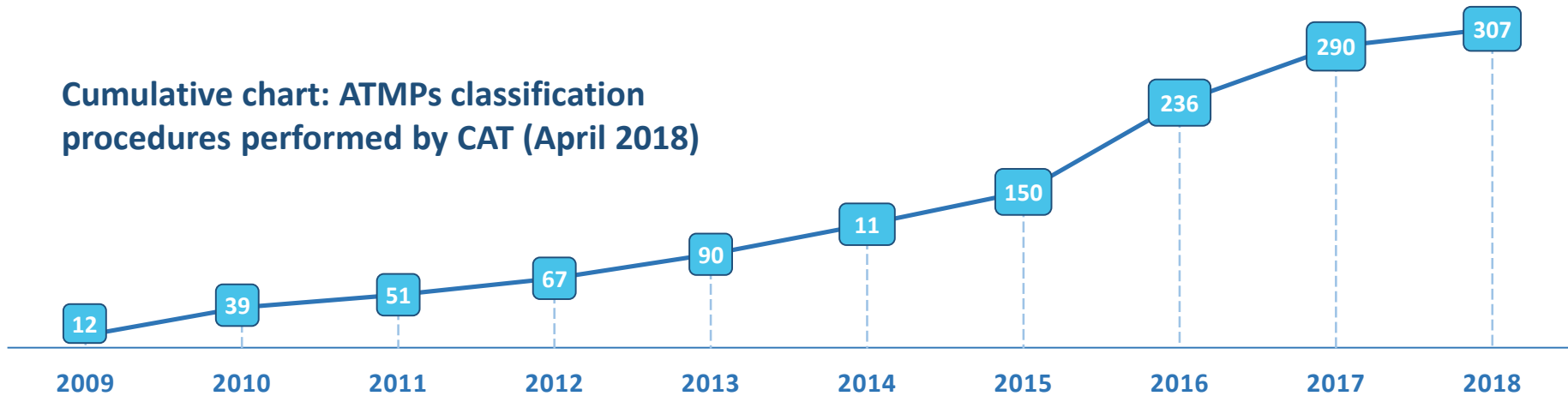
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ATMPs: Gaining Momentum

- 21 ATMPs Marketing Authorization application submissions (until 2018)
- 12 ATMPs received marketing authorization
- 8 Currently marketed ATMPs in the EU
- 3 ATMPs Authorized in 2018
- 2 Authorized ATMPs with PRIME designation

Cumulative chart: ATMPs classification procedures performed by CAT (April 2018)





Implementation hurdles for ATMPs

Small scattered
patient populations



Insufficient Evidence
& Under-Powered Trials



Training of Healthcare
Providers



Complex administration
procedure



Pricing &
Reimbursement



Severe/Unexpected
Side-Effects



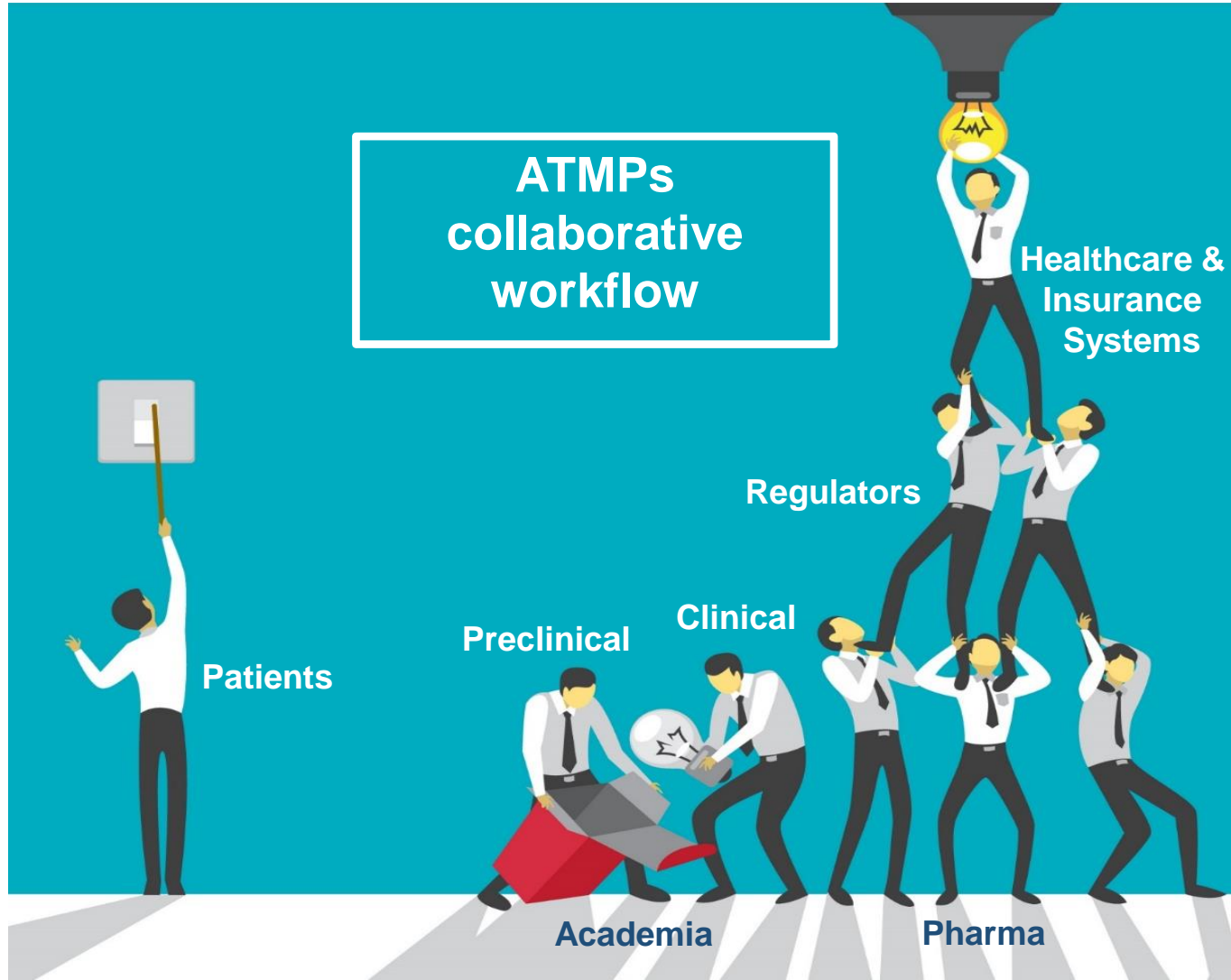
Unique Manufacturing
Processes



Complex supply chain



Abou-El-Enein et.al. Cell Stem Cell, 2016



The patient will always be the key to success of ATMPs



SOLUTIONS

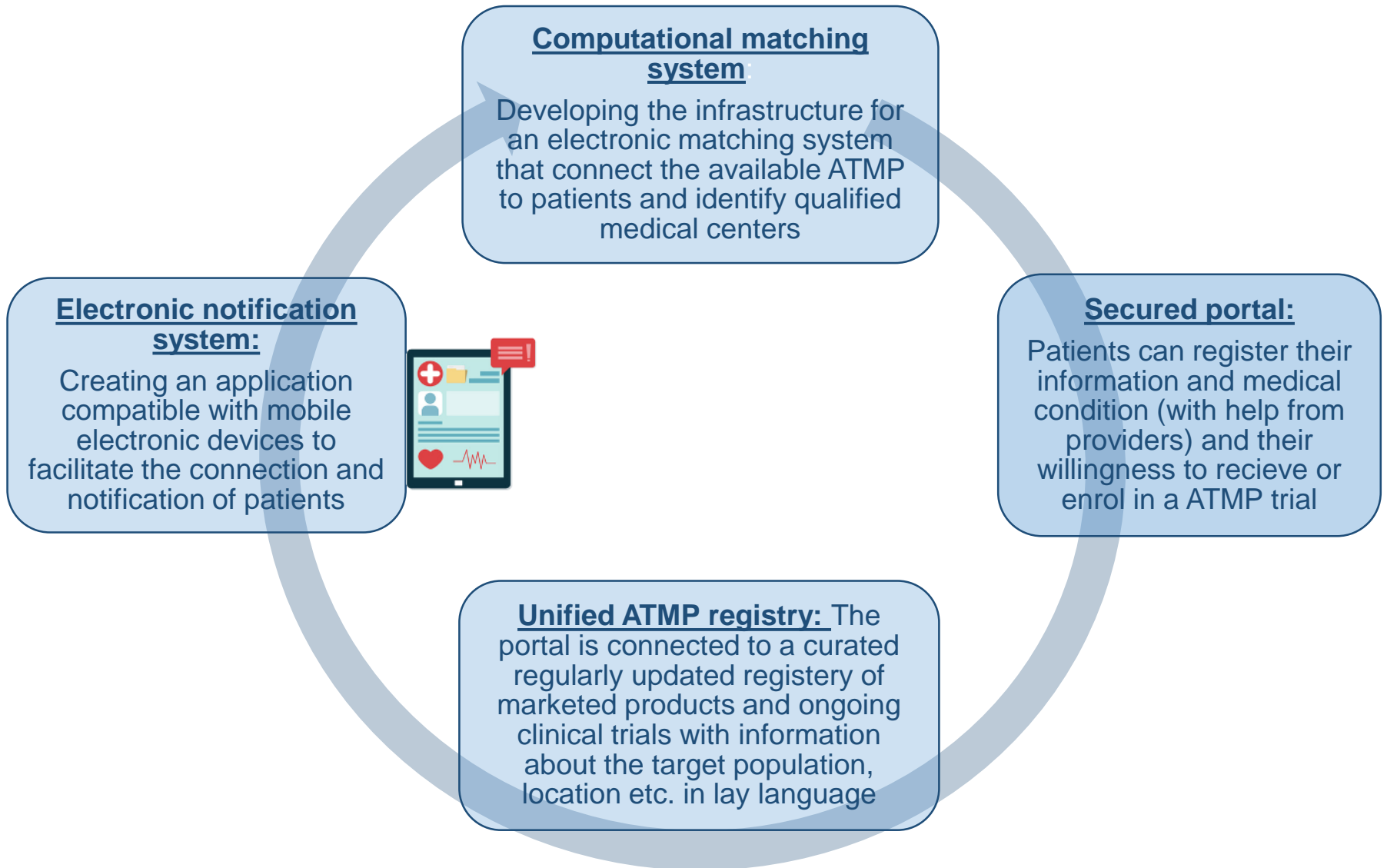
ATMP Matching Program:

The main goal is linking the right patient to either

- ❑ marketed ATMPs and
- ❑ experimental ATMPs (being tested in regulated clinical trials)

at qualified medical centers/trial sites based in the EU







A delivery platform for marketed and experimental ATMPs: can help in

More patients receiving the therapies they are in desperate need for

- Increasing patient enrollment in the clinical trial which will improve the quality of evidences generated
- Increasing collaboration between pharma & healthcare systems to set up more qualified products administration and follow-up units
- Promoting closer interaction between developers, patients, advocacy groups and insurance bodies to address reimbursement challenges
- Becoming a platform for offering training programs for healthcare providers
- Tackling and limiting the exposure to unproven cell-based treatments (consult patients and their families)
- Increasing awareness among patients and patient advocacy groups on the benefits and risks of cell therapies



Similar Initiatives “but not the same”



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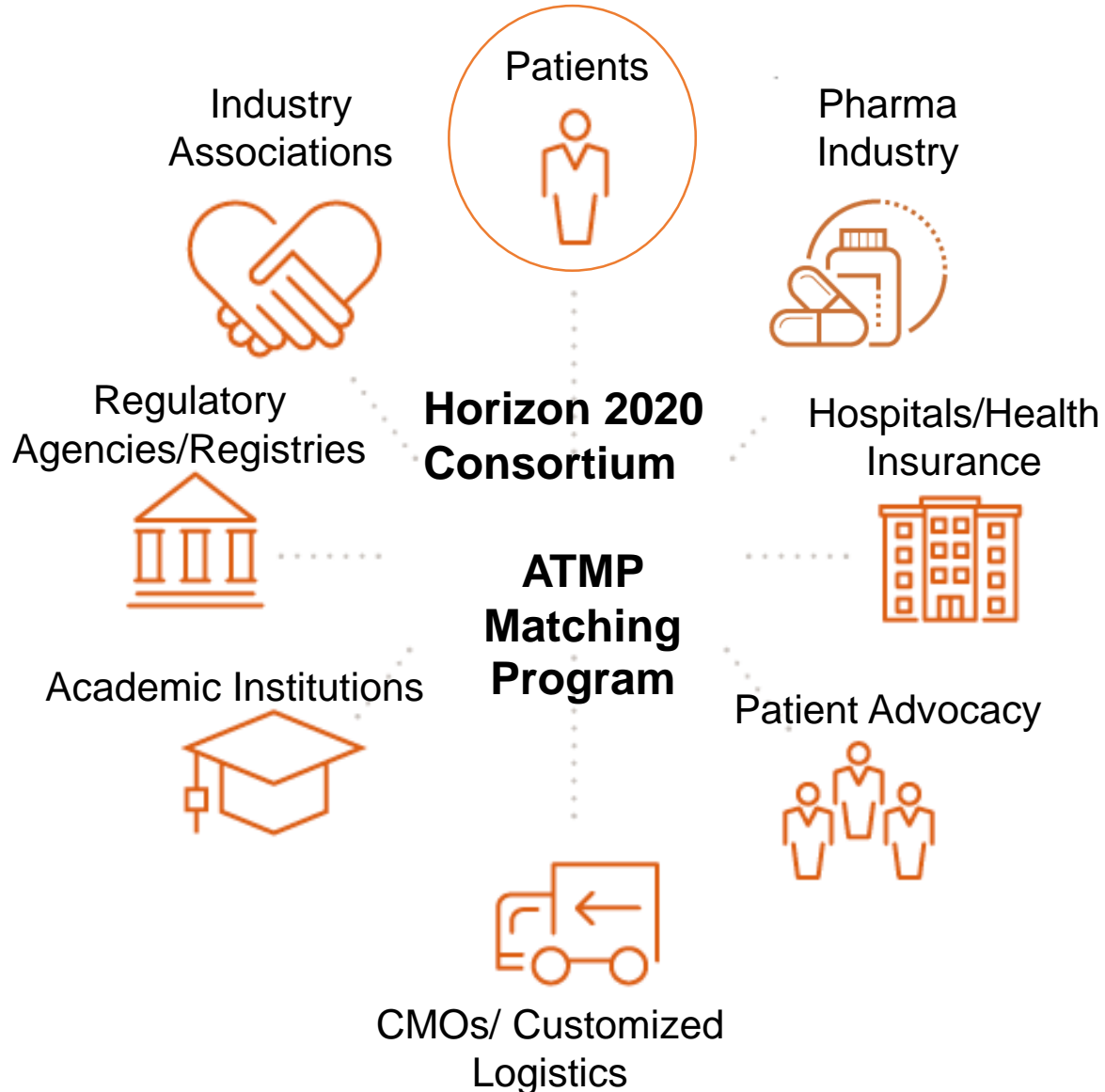


Widening the Reach of Drug Development

Understand Expanded Access in 8 Minutes



Next Steps: Involvement of all Stakeholders





Patient engagement is unavoidable: patients are no longer end users when it comes to individualized therapies

All stakeholders need to collaborate for the successful delivery of ATMPs to patients in need in Europe and worldwide

A European ATMP Matching Program could help increasing access and optimizing delivery of ATMPs to patients



*“If everyone is moving forward together,
then success takes care of itself.”*

Henry Ford

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